

A woman with blonde hair is holding a young child with dark hair. They are outdoors, with a clear blue sky and some greenery in the background. The woman is looking off to the side with a thoughtful expression, while the child looks directly at the camera.

# From bench to bedside through industry collaborations

Annamaria Merico, Head of Technology Transfer Telethon Foundation

FONDAZIONE





# Telethon Mission & Vision

- **Fondazione Telethon** is a major Italian biomedical charity focused on genetic diseases
- **Founded in 1990** at the behest of a group of **patients**
- Supported through **fundraising**

## OUR MISSION

*Advance biomedical research  
towards the cure of genetic diseases*

**592,5 M€** research investment  
**14.212** papers published

**1.640** PIs awarded  
**584** genetic diseases studied

## OUR VISION

*Convert the results of excellent, selected and sustained research  
into available therapies*

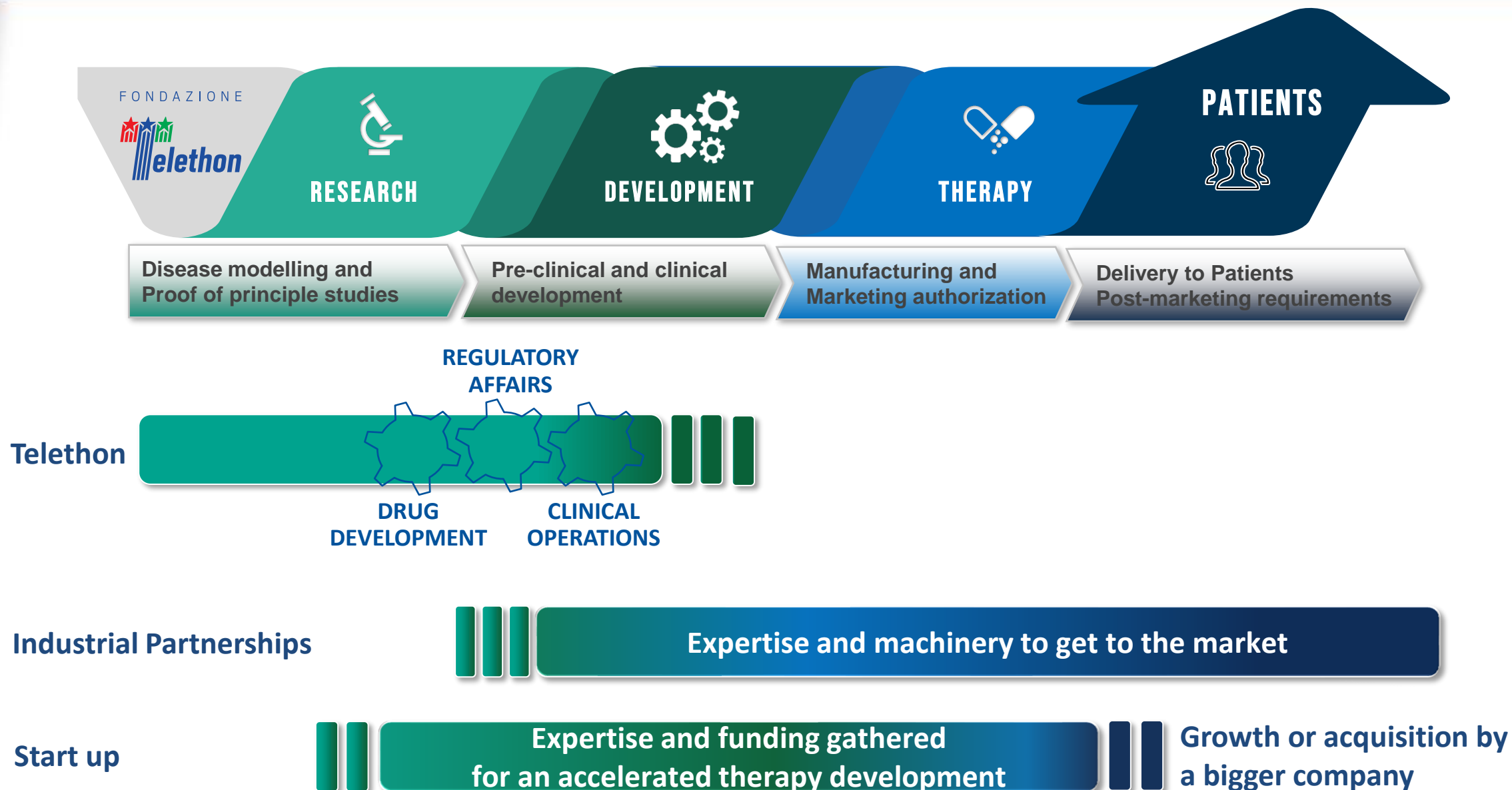
**130** patients treated

**2 Therapies approved**



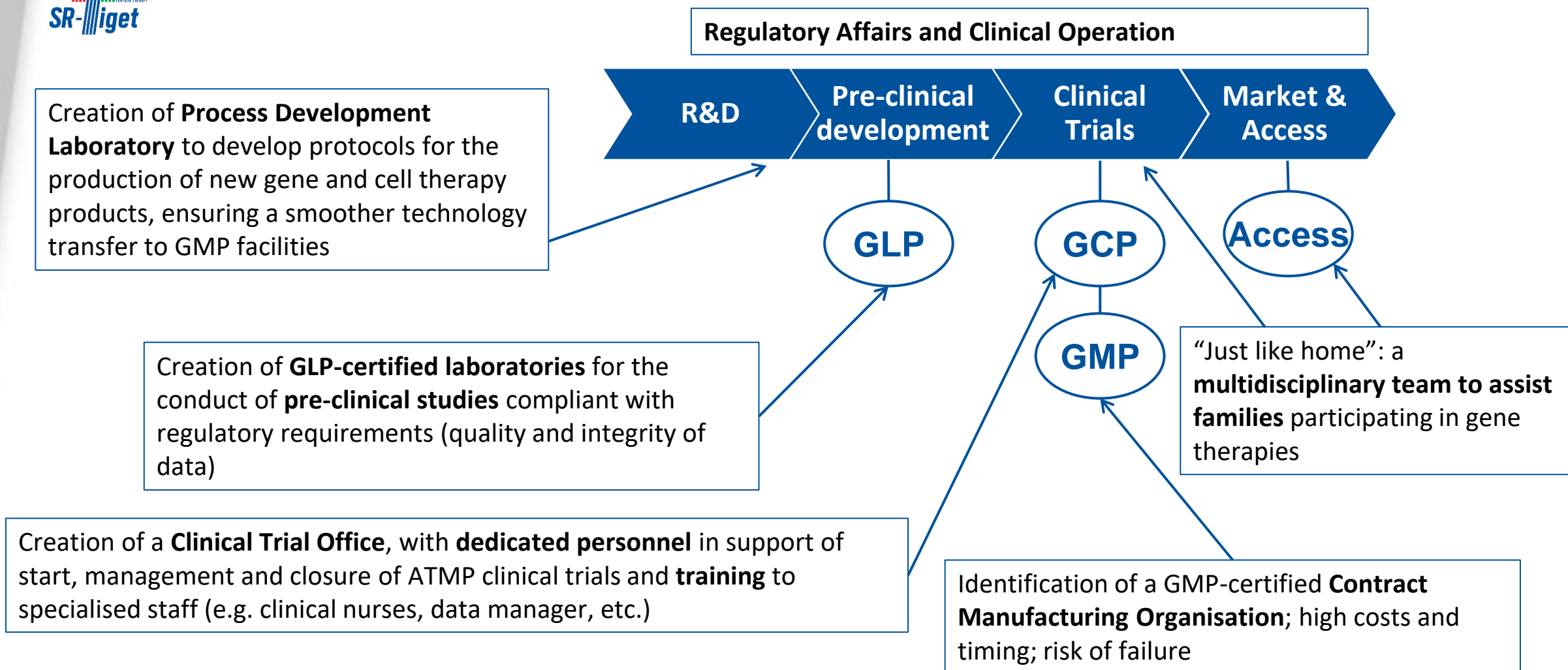


# Teaming up with industry and investors





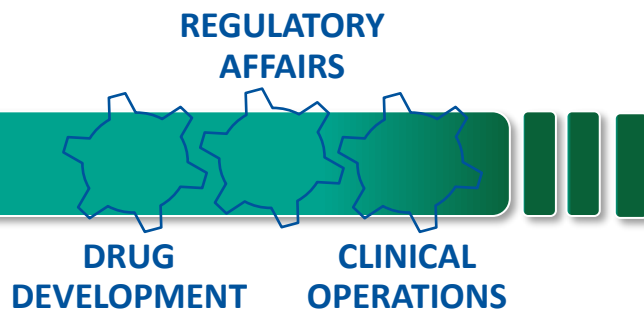
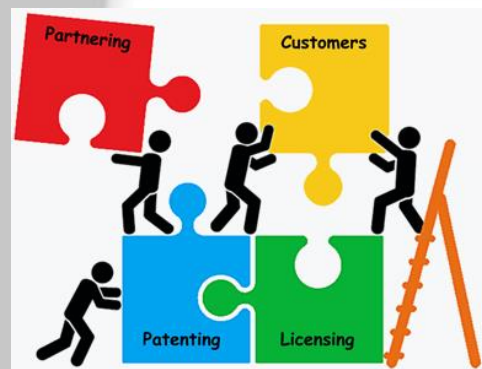
# Telethon supports derisking activities to accelerate development



None of these actions are typical of a charity



# Our Model



Industrial Partnerships



Start up



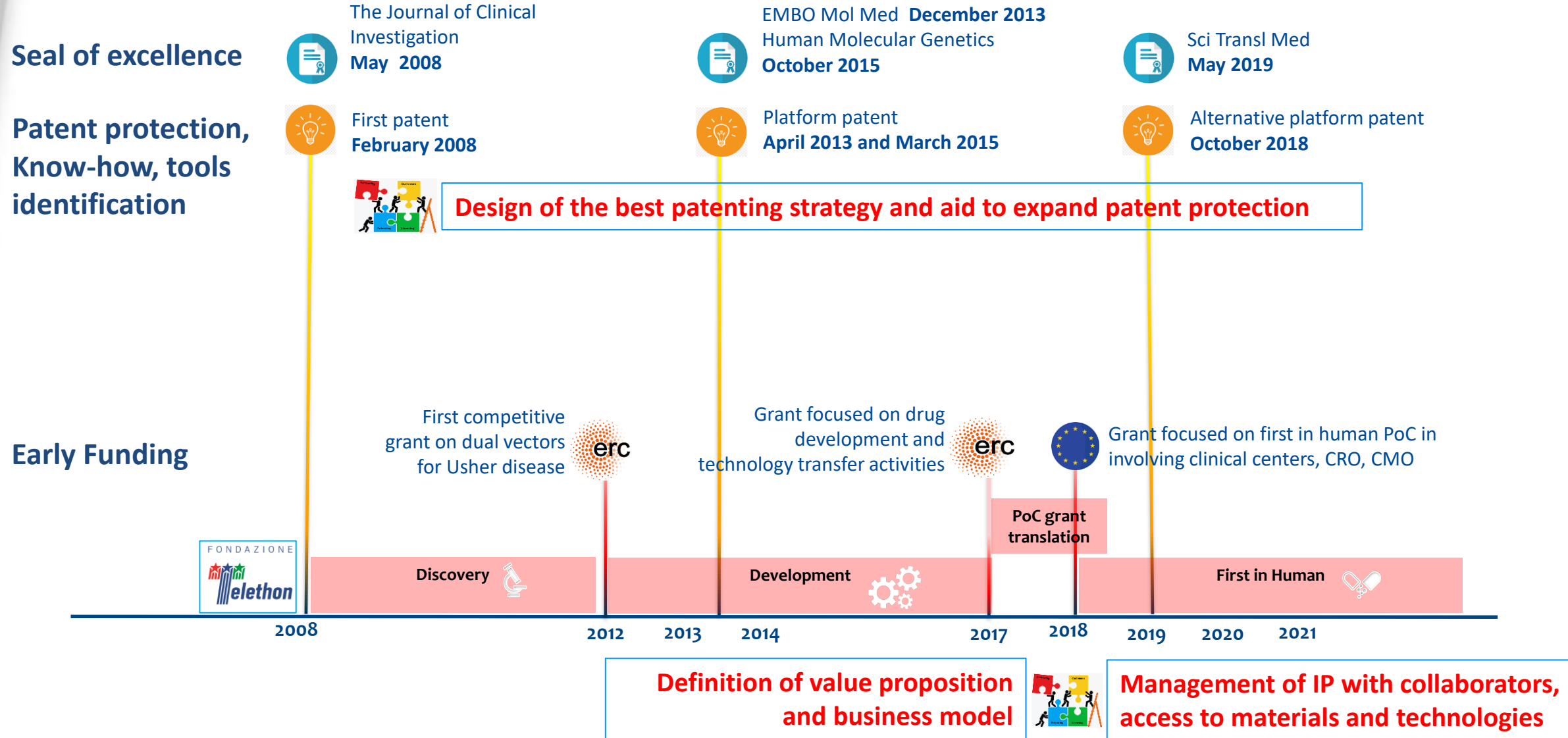
Growth or acquisition by a bigger company



# A long journey guided by industry development vision



## Therapy for rare eye diseases





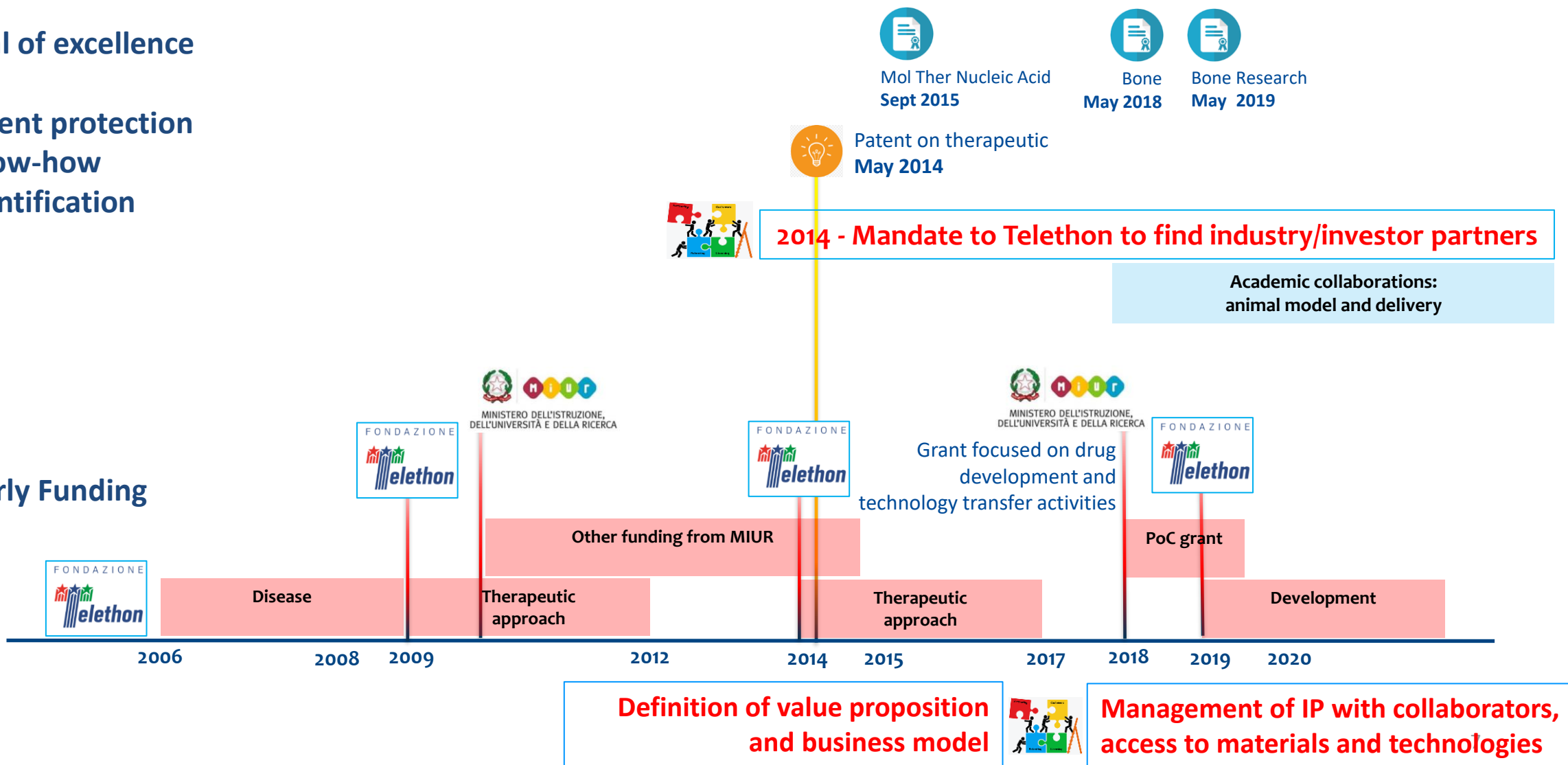
# The partnership between Scientist and TTO is critical

## Therapy for rare bone diseases

Seal of excellence

Patent protection  
Know-how  
identification

Early Funding



# Why early management of IP is relevant?

It relates to research results (data and materials):

1. Is your Institution the owner of the results?
2. Can you freely use the results?
3. Can you freely publish the results?
4. Can you use the results also in collaboration with others?
5. Can your Institution exploit the results in industrial partnerships and startup?

TTO tries to get this degree of freedom!

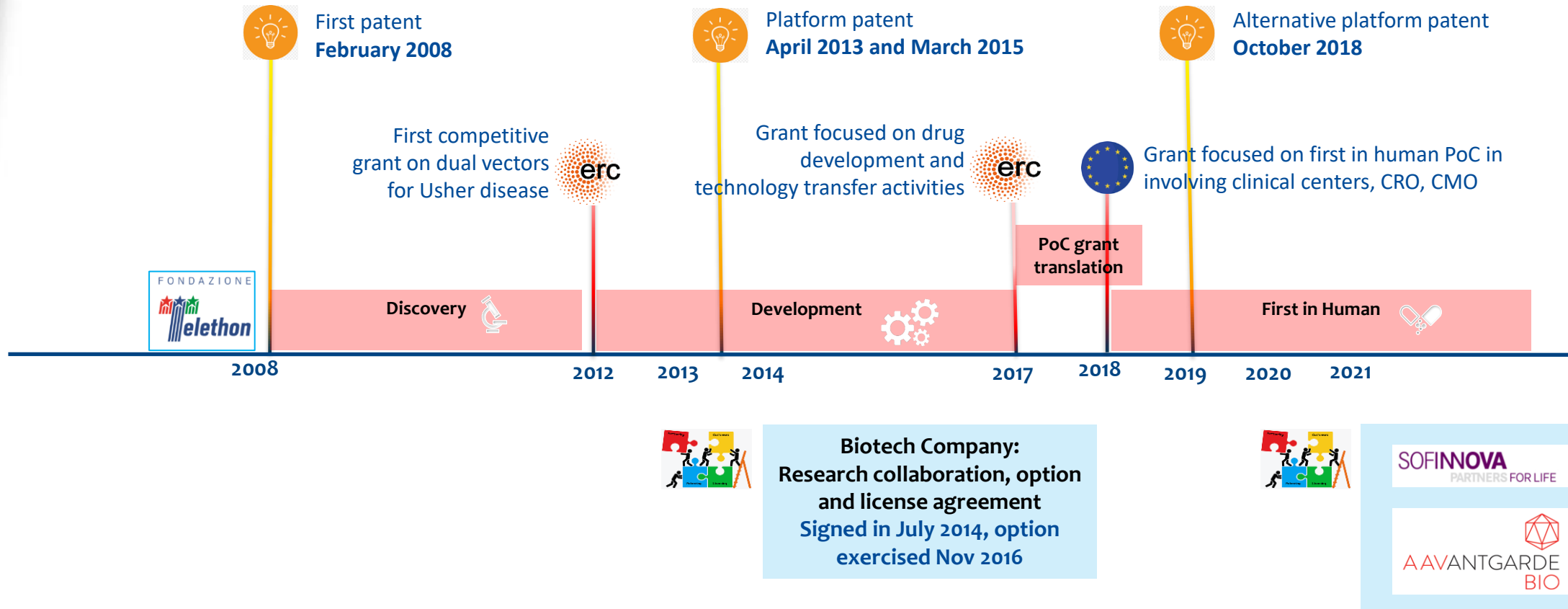




# Partnerships always trigger a learning process



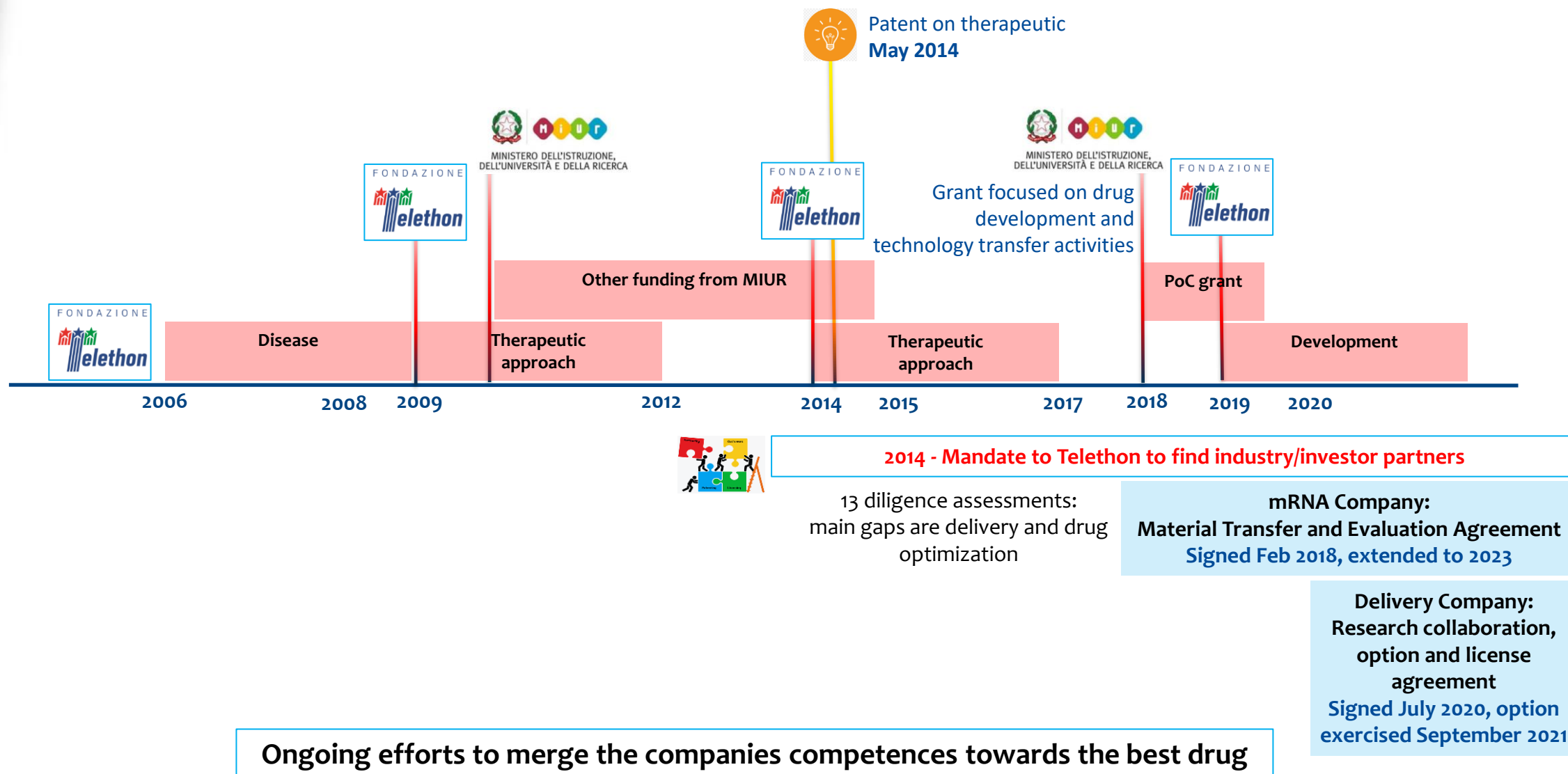
## Therapy for rare eye diseases





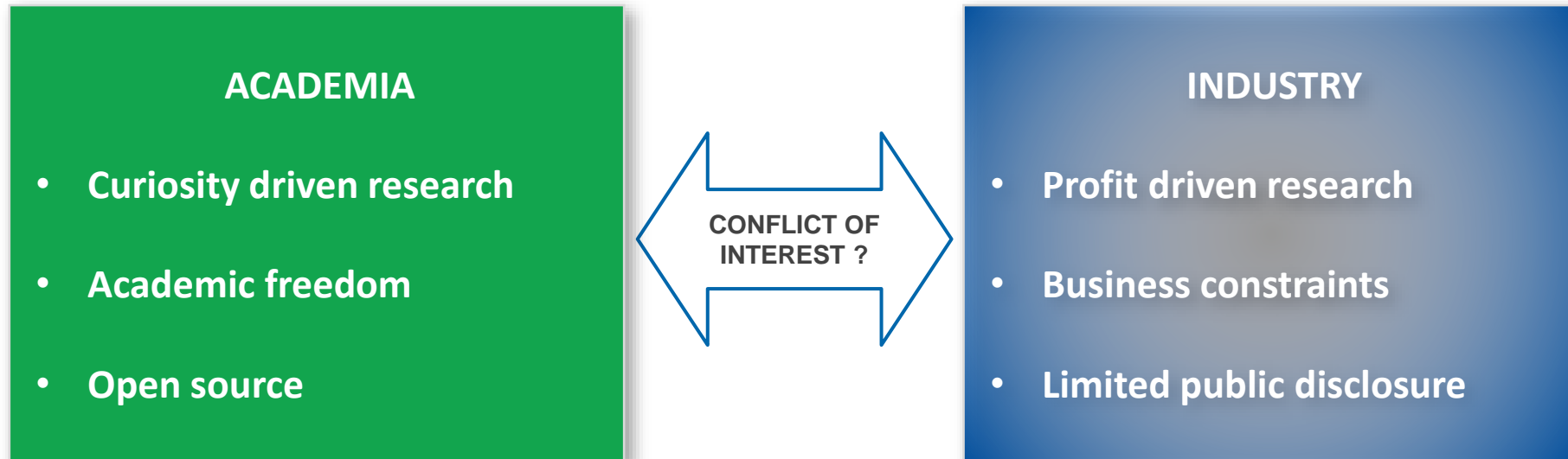
# Technology transfer is not a one-shot activity

## Therapy for rare bone diseases





# Is it possible to partner with industry?



# The pillars of technology transfer

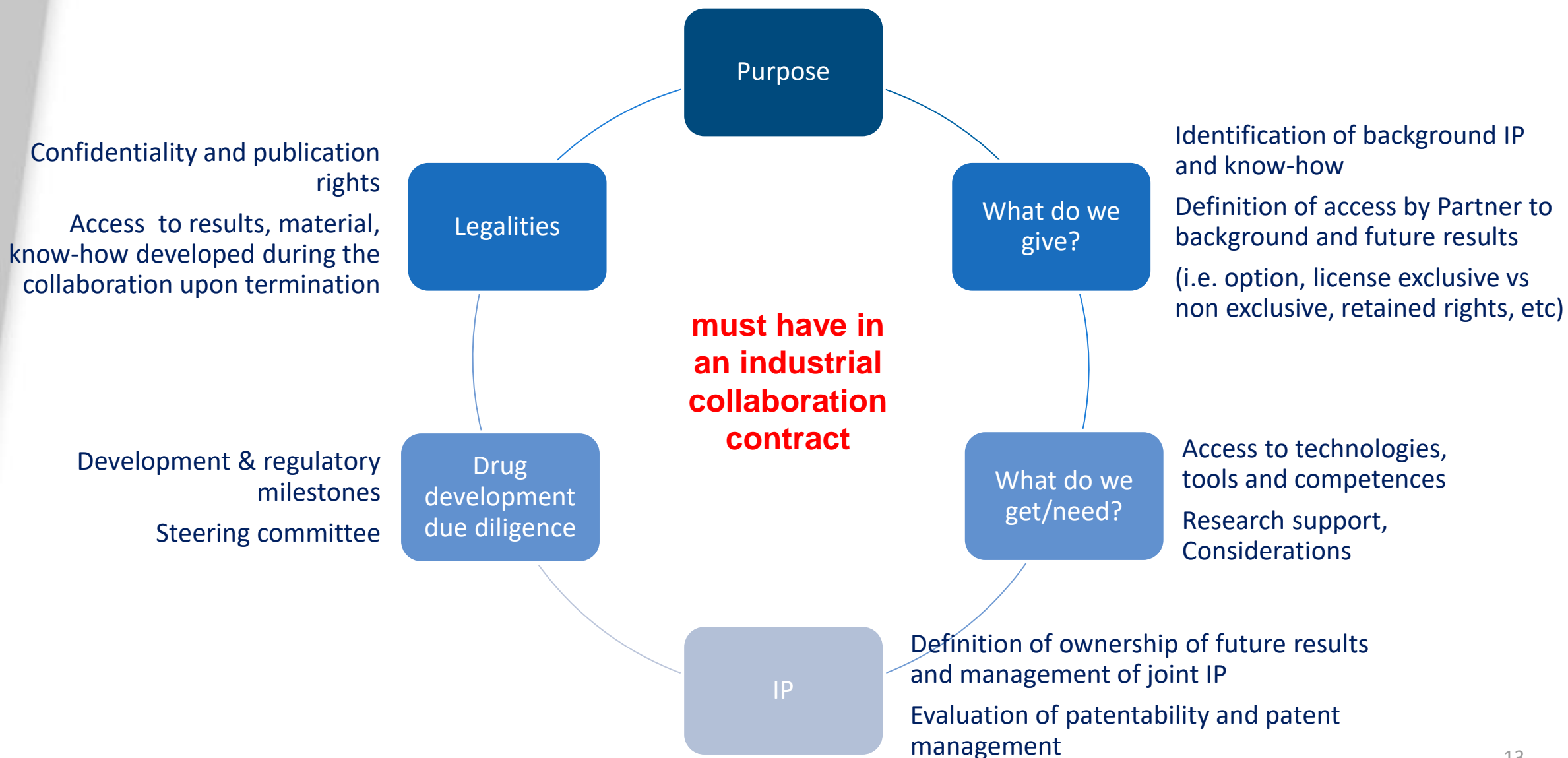
TTO should work in **strict cooperation with scientists**, acknowledging their crucial roles in research development

- **Safeguard research independence**
- **Retain intellectual property rights** to safeguard opportunities of development
- In case the partner fails to pursue therapy development, **revert both IP and co-developed results back to FT**
- **Partnerships should provide** adequate **research funding** support
- **Partnerships should** supply **additional funding** through development and commercial milestones and royalties





# Scientist can contribute to negotiation



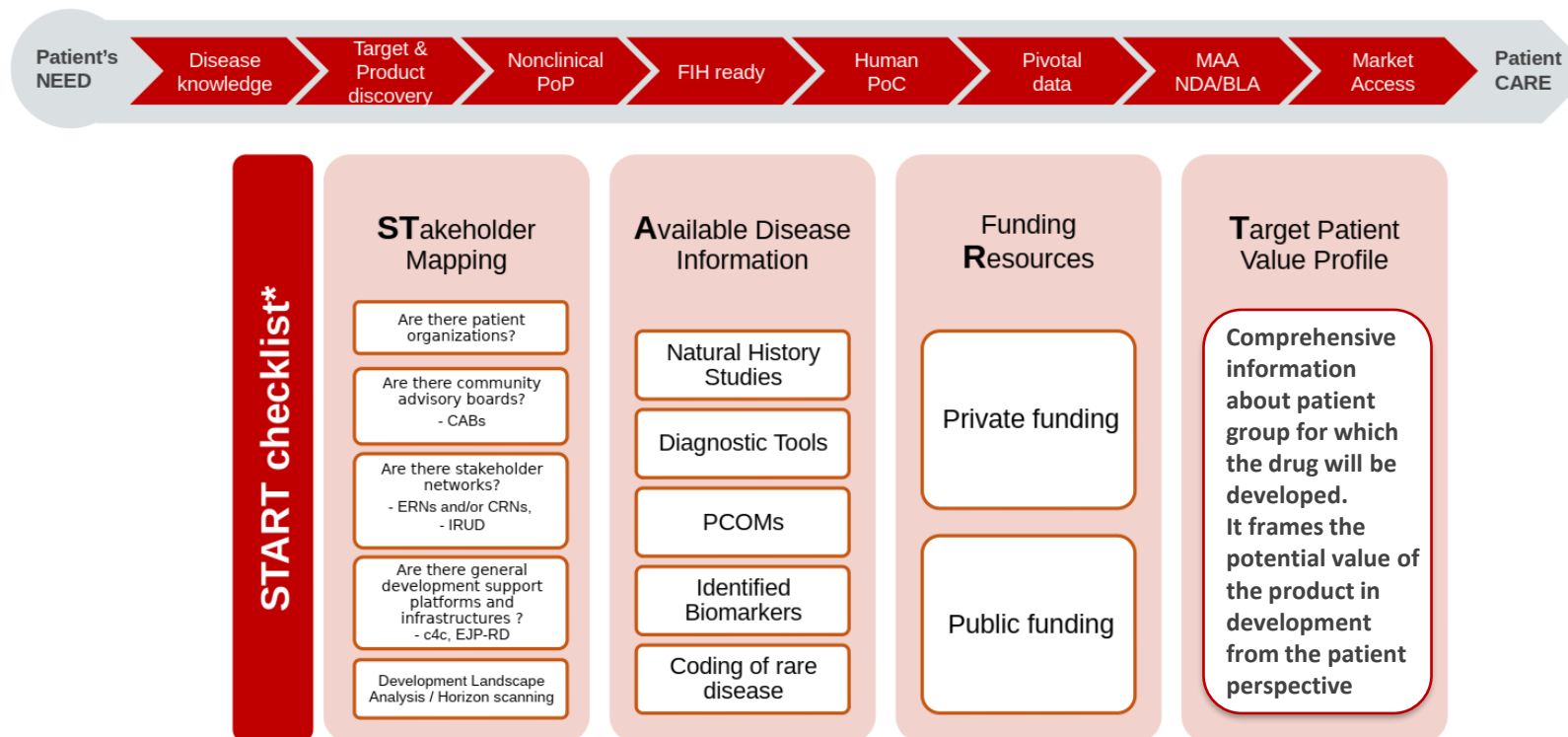


# IRDiRC Orphan Drug Development Guidebook: now available interactive!



A simple patient-focused **guidebook** that describes the available tools, incentives, resources and practices specific for developing traditional and innovative drugs/therapies for **rare disease** indications and how to best use them.

## How to start your orphan drug development



\* If this information not available, the first step is to build up the stakeholder mapping, and then generate data to the best of the possibilities.

THANKS!

FONDAZIONE

